What is Cognate BioServices?

Contract/consulting services provider for the field of cell-based therapeutic products

- Preclinical
- cGMP (current Good Manufacturing Practice) manufacturing of cells
  - Preclinical work
  - Clinical trials
- Quality control/quality assurance

Cognate BioServices, Inc.

- Manufacturing process development and scale-up

Facility

- cGMP stem cell manufacturing facility in Sunnyvale, CA—~10,000 sq.ft. manufacturing suite
  - Issued CA Dept. of Health Services, Food and Drug Branch manufacturing license
  - U.S. FDA approved clinical manufacturing
- Regulatory consulting

Cognate BioServices, Inc.

- Staff involved in obtaining -16 FDA Investigational New Drug (IND) approvals--for human clinical trials with stem cell or cell-based therapeutic products

- Many years of collective experience with translational "productization" of stem cells and cell-based therapeutics

- The Company specializes in "How to get from the bench to the bedside with a cell therapy product."

Stem Cell Development Challenges

- Cell sourcing, banking and characterization
  - Characterization and quality control
  - Documentation and traceability
  - Storage/stability of banked cells
- Laboratory scale to clinical and commercial scale process (cGMP process scale-up) needs to be part of the translational research
  - Reproducible, reliable, robust process
  - Clinical/commercial scale

Stem Cell Development Challenges

- Suitable cGMP manfacturing
  - Appropriate manufacturing facilities available for use
    - Multi-use & multi-suite--flexible mfg. capabilities
  - Trained manufacturing, quality control and quality assurance staff for stem cell-based therapeutics
  - Use of suitable cGMP manufacturing materials, methods and documentation
- Suitable quality control and quality assurance procedures
  - Demonstrate safety (sterility, endotoxin, tumorigenicity)
  - Demonstrate consistency (purity & potency)
  - Characterization and QC methods development

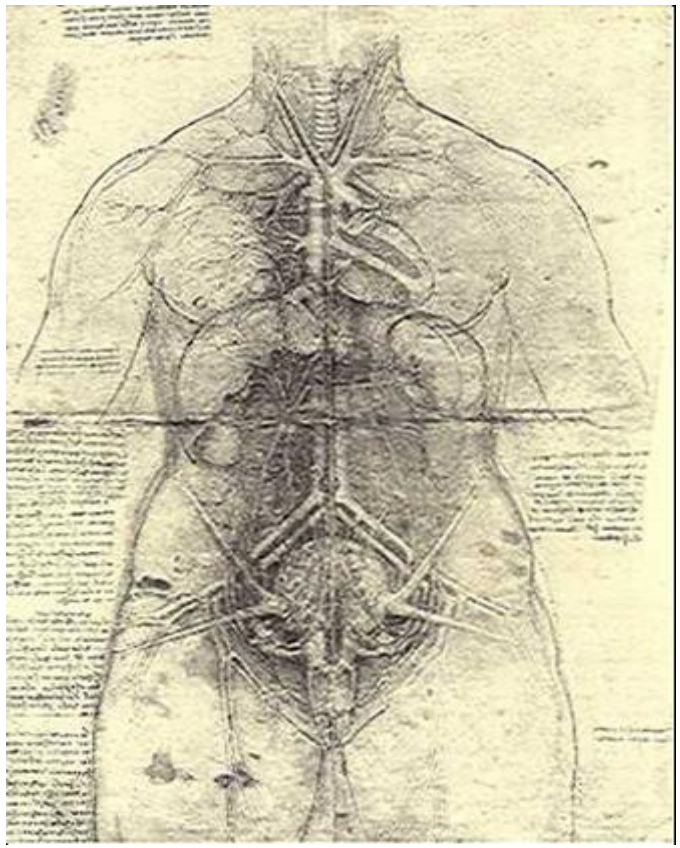
Stem Cell Development Challenges

- Storage & distribution logistics
  - Often overlooked but critical to widespread clinical application/commercialization
- Consulting resources for education/training
  - Develop a resource base in California for CIRM funded projects
  - Training on cGXPs (where x=laboratory, tissue, manufacturing and clinical)
  - Encourage scientists and clinicians to draw on the resources early and often to avoid expensive mistakes
  - An ounce of prevention may be worth millions in savings on a misdirected project in this area

Other Considerations

- Guidelines and resources for stem cell product standardization? Lack of common standards?
  - Comparability of SC or SC derived product composition by groups
- New/improved tools required?
  - Analytical Tools
    - Trafficking/tracking of cells in preclinical studies
    - QC assays for potency, drug substance determination
    - Improved cryoprotectants
    - Serum free/defined culture media
- Community success or failure—we all share in either outcome

Stem Cell-based Therapeutics and Regenerative Medicine



How do we work together to expedite the translational research and help patients?

...The Race is On!